

# Pioneering the next generation of drug discovery for rare diseases

**Dr Tim Guilliams** 

Co-Founder and CEO of Healx

### Healx is an Al-powered biotech developing treatments for rare diseases at an unprecedented scale



### Inspired by the patients and families living with rare diseases



Nick is not alone

## 7000

## 400 mil.

## 95%

rare diseases

people affected worldwide

without an approved treatment



But traditional drug discovery is broken

## > 10 years

> \$2 bil.



to bring a drug to market

in R&D and market costs

treatments fail to make it to market



#### Why is traditional drug discovery broken?

### 66

#### One disease, one target, one drug

- the old target-based paradigm was overly simple for complex diseases and broke the industry. We're changing that.

Dr David Brown Co-founder & Chairman, Healx Former Global Head of Drug Discovery at Roche and co-inventor of Viagra

### Healx is pioneering the next generation of drug discovery



### We are redefining and accelerating the drug discovery process





Faster

 $\bigcirc$ 

Higher success rate



More capital efficient

## Supported by a three-way therapeutic strategy with multiple shots on goal to increase likelihood of clinical success

#### Drug redevelopment



Allows us to make the most of existing safety and efficacy data to accelerate treatment development to the clinic

### Molecule enhancement



Derisked NCE approach by starting from known drug prediction (or combination) and enhance its molecular properties to desired profile

#### Combination therapies

Higher chance of efficacy by targeting multiple modes of action and different disease pathways

#### heal×

### It works. In a fraction of the time and cost, Healx has built a large therapeutic portfolio in rare disease.

Disease Area	Indication	AI Discovery	Preclinical	Clinical Planning	Phase 1/2a
Neurodevelopmental	Fragile X Syndrome			OV/d T	IND approved
	Undisclosed				
	Angelman syndrome				
Renal and Liver	ADPKD				
	ARPKD				
	Undisclosed				
	Undisclosed				
	Undisclosed				
Oncology	NF1: cutaneous neurofibroma (cNF)				
	NF1: plexiform neurofibroma (pNF)				
Neuromuscular	FSHD				
	Undisclosed				
Inflammatory	Chronic pancreatitis				
Bone	Undisclosed				
Multiple areas	6 undisclosed rare diseases				
Indication expansion partnership (2x)		ONO			



### We're taking our first programme to clinical trial this year



### **Forbes**

EDITORS' PICK | Oct 20, 2021, 05:00am EDT

Clinical Trials Expand For Al-Designed Drug To Treat Fragile X Syndrome

TechCrunch

#### UK startup Healx to begin clinical trial on repositioned drug for Fragile X syndrome

Emma Betuel @emmakbetuel / 2:46 PM GMT+1 • October 20, 2021

Comment

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### About Fragile X syndrome and our partnership with FRAXA





6 The Healx partnership has been nothing short of amazing. In less than two years together, we were able to deliver decades-worth of drug discovery, and now we're taking those discoveries to the clinic.

Michael Tranfaglia Medical Director and Co-Founder FRAXA Research Foundation

### More about our technology platform, Healnet



## Data-driven disease ranking and selection to increase our probability of clinical success



Clinical endpoints & biomarkers

## We apply omic- and graph-based prediction methods to find new disease mechanisms and treatments



### Our parallel approach to treatment prediction and ranking



- PRISM/DWPC
- DRAM/PASDA
- KBC/AnyBurl



## Identifying new disease mechanisms and drug mode of action with 'Evidence Chain' algorithms



## Long term vision: towards Massively Parallel treatment predictions and testing



#### Get in touch

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Dr Tim Guilliams and Dr David Brown, Healx's Co-Founders

